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**Motor neuron diseases: Finding cures through basic, translational, and clinical collaboration**

**Grant Award Details**

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Motor neuron diseases: Finding cures through basic, translational, and clinical collaboration

**Grant Type:** Disease Team Planning

**Grant Number:** DT1-00659

**Investigator:**

<b>Name:</b>	Samuel Pfaff
<b>Institution:</b>	Salk Institute for Biological Studies
<b>Type:</b>	PI

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**Award Value:** \$54,016

**Status:** Closed

**Grant Application Details**

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**Public Abstract:**

Spinal Muscular Atrophy (SMA) and Amyotrophic Lateral Sclerosis (ALS) are motor neuron diseases. Motor neurons control the voluntary muscles that are used for activities such as crawling, walking, head and neck control, and swallowing; and sadly there are no known cures for motor neuron diseases at this time. SMA is a genetically inherited disorder and about 1 in 40 people are carriers. SMA symptoms typically become apparent soon after birth and is a devastating childhood disorder that is relatively common and affects approximately 1 in 6000 babies. A mutation in the SMN1 gene has been identified as being responsible for SMA and researchers have developed excellent animal models to investigate the cellular and molecular features of the disorder. Although ALS is distinct from SMA and does not typically begin to manifest itself until 30-40 years of age, it too is a motor neuron disorder which affects an estimated 100,000 Americans. 3% of ALS cases are due to mutations in the SOD1 gene, and like SMA excellent animal models have been created by researchers to study the disease and test ideas for treatment. The potential to use stem cells to help characterize drugs and test cell replacement strategies is extremely exciting for motor neuron diseases because of two recent findings: the ability to efficiently convert ES cells (both mouse and human) into cholinergic motor neurons and the promise of deriving ES cells from many different cellular sources. Although much work remains to be completed, science is well positioned to begin translating its findings into beneficial treatments.

This grant will be used to hold a workshop among experts in the field of motor neuron diseases to identify the most promising approaches for treating a particular motor neuron disease. Several criteria will be used in this planning process, including: (1) evaluating where the state-of-the-art science is at that moment, (2) considering current approaches being tested and avoiding unnecessary overlap, (3) finding the "lowest hanging fruit" and focusing efforts primarily on those objectives, (4) taking optimal advantage of the immense human and physical resources available in the [REDACTED] community by combining the efforts of researchers and clinicians located at the [REDACTED], [REDACTED], [REDACTED], and [REDACTED]. The deliverable from this grant will be to create a report that outlines recommendations for using stem cells as a tool or a treatment for curing a motor neuron disease. This plan will include recommendations for the scientific direction, as well as management and administration suggestions to ensure the highest possibility of success.

**Statement of Benefit to California:**

The primary goal of this collaborative endeavor is to develop highly efficacious treatments and cures for motor neuron diseases which affect the lives of thousands of Californians. This should not only markedly improve the longevity and quality of life for affected individuals, but will help to address the immense burden and expense of providing care to individuals with motor neuron diseases. We also anticipate several indirect benefits to the citizens of California, including the creation of intellectual property that can help to create new jobs in the technology sector. In addition, it is likely that the process identified for treating motor neuron diseases can likewise serve as a template for creating strategies that will ultimately yield treatments for more challenging neurological diseases and disorders including spinal cord injury, Parkinson's, and Alzheimer's disease, for example.

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